

Dr. Siegel Gene Therapy

Ground Zero Pharmaceuticals, Inc.

***Innovative Product Discovery
and Development Consulting,
Company- Building and Joint
Ventures in Pharmaceuticals,
Biologics and Medical Devices***

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Leveraging - Collaborating With Stakeholders

Assuring Industry Compliance with Safety Regulations - Gene therapy, Human Cellular and Tissue Based Products

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Presented at Stanford University

March 23, 2000

Regulatory Concerns

- ▶ 21 CFR Parts 50 and 56
- ▶ 21 CFR 312 *et seq.*
- ▶ 21 CFR 314 and 600 *et seq.*
- ▶ Disbarment of Investigators
- ▶ NIH/FDA Conflicts
- ▶ Spillover of Effects to other Programs
- ▶ Impact on the System of Voluntary Compliance

Clinical Development Issues

- ▶ Time and Financial Constraints
- ▶ “Founders Disease”
- ▶ Protection of Subjects and Patients
- ▶ Cutting Edge Viral Vector Research
- ▶ The Need for Manufacturing Controls for Cell Substrates, Cellular and Tissue - based Products
- ▶ “Scientist vs. Clinician” Identification

Sponsor Responsibility

- ▶ Regulatory Requirements
- ▶ Protection of Patients
- ▶ Financial Accountability/Research Success
- ▶ Communication of Results

Some Solutions

- ▶ 3rd Party Review of Research Programs, Outside of Government
- ▶ 3rd Party Review of Toxicity of Viral Vector Research Intended for Human Gene Therapy
- ▶ Manufacturing Certification/3rd Party Review for Cell Substrate Facilities and QC/Safety for Cellular and Human Tissue-Based Products

Critical Issues

- ◆ Need for Faster, More Cost-Effective Product Development
- ◆ Requires Collaboration of FDA, Sponsor, Investigator(s), Others
- ◆ Need for Faster and More Complete Reporting of Safety Data
- ◆ Assure Communication Without Fear
- ◆ Establish Public Policy

Execution

- ▶ Use State Government, Corporate Expert Committees to Certify Technology and Facilities
- ▶ Ensure Minimal Conflict of Interest
- ▶ Protect Intellectual Property
- ▶ Provide Joint Training of All Investigators and Provide Certification
- ▶ Educate Investigators and Patients to the Risk/Benefits of Research

Philosophy

“Lack of Timely Decision Making, in a Thoughtful and Logical Manner, is the Singular Cause of Delays, Wasted Effort and Needless Expenditures in Product Development”

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